

What is claimed is:

- Sub B1* > 1. A method for predicting the effect of a drug candidate compound, comprising:
- a) modulating, by somatic gene transfer, expression of a selected protein in selected cells; and
 - b) analyzing the result of expression of the protein to thereby predict the effect of the drug candidate compound.
2. The method of claim 1 wherein the result of the protein expression is analyzed to identify a molecular target of the drug candidate compound.
3. The method of claim 1 wherein the result of the protein expression simulates the predicted pharmacological effect of the drug candidate compound.
4. The method of claim 1 wherein the result of the protein expression alters function of the cells.
5. The method of claim 1 wherein a function of the cells is suppressed by the protein expression.
6. The method of claim 1 wherein a function of the cells is induced by the protein expression.
- Sub B2* > 7. A method for detecting a potential drug target protein, comprising modulating, by somatic gene transfer, expression of the target protein in selected cells, and analyzing the result of expression of the target protein.
8. The method of claim 7 wherein expression of the target protein is increased following the somatic gene transfer.
9. The method of claim 8, wherein the increase in expression is achieved by operably linking a gene encoding the target protein to an inducible or viral promoter.
10. The method of claim 7 wherein expression of the target protein is inhibited following the somatic gene transfer.
11. The method of claim 10 wherein expression of the target protein is inhibited by transfer of a gene truncated relative to a corresponding native gene.

12. The method of claim 11, wherein the truncation is a contiguous or non-contiguous deletion of the transferred gene.

13. The method of claim 10, wherein expression of the target protein is inhibited by transfer of a gene encoding one or more amino acid substitutions relative to a corresponding native protein.

Sub 8 > 14. The method of claim 7, wherein the target protein is capable of specifically forming a binding complex with at least one other protein.

15. The method of claim 14, wherein expression of the target protein is sufficient to produce a dominant negative mutation that reduces or blocks function of the binding complex.

16. The method of claim 7, wherein the potential drug target protein is an ion channel protein, the selected cells are capable of producing an ion current, and the analysis comprises measuring the ion current produced by the selected cells.

17. A method of detecting an ion channel capable of serving as a drug target protein, the method comprising:

- a) providing a population of somatic cells capable of producing an ion current from a recombinant nucleic acid segment encoding an ion channel protein;
- b) modifying the nucleic acid segment sufficient to alter the ion current produced by the encoded ion channel protein;
- c) transferring the modified nucleic acid segment into the somatic cells under somatic cell gene transfer conditions which allow expression of the encoded ion channel and production of an altered ion current;
- d) detecting the altered ion current; and
- e) correlating the altered ion current to the capacity of the ion channel protein to serve as the drug target protein.

18. The method of claim 17 wherein expression of the ion channel protein is increased following the somatic gene transfer.

19. The method of claim 17 wherein expression of the target protein is inhibited following the somatic gene transfer.

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20. The method of claim 19 wherein expression of the target protein is inhibited by transfer of a gene truncated relative to a corresponding native gene.

21. The method of claim 20, wherein the truncation is a contiguous or non-contiguous deletion of the transferred gene.

22. The method of claim 19, wherein expression of the target protein is inhibited by transfer of a gene encoding a modified protein comprising one or more amino acid substitutions relative to a corresponding native protein.

23. The method of claim 19, wherein the target protein is capable of forming a binding complex with at least one other protein.

24. The method of claim 23, wherein expression of the target protein is sufficient to produce a dominant negative protein that reduces or blocks function of the binding complex.

25. A method of reproducing a cardiac arrhythmia phenotype in a population of cultured cells, the method comprising:

- a) providing a population of cultured somatic cells capable of producing an ion current from a recombinant nucleic acid segment encoding an ion channel protein;
- b) modifying the nucleic acid segment sufficient to alter the ion current produced by the encoded ion channel protein;
- c) transferring the modified nucleic acid segment into the cells under conditions which allow expression of the encoded ion channel and production of the altered ion current;
- d) detecting the altered ion current; and
- e) correlating the altered ion current to the capacity of the ion channel to serve as the therapeutic target.

26. A method of reproducing a cardiac arrhythmia phenotype in a population of cultured cells, the method comprising:

- a) providing a population of cultured somatic cells capable of producing an ion current from a recombinant nucleic acid segment encoding an ion channel protein;

- b) modifying the nucleic acid segment sufficient to alter the ion current produced by the encoded ion channel protein;
- c) transferring the modified nucleic acid segment into the cells under conditions which allow expression of the encoded ion channel and production of the altered ion current; and
- d) detecting the altered ion current sufficient to reproduce the mammalian cardiac arrhythmia in the cultured cells.

27. The method of claim 25 or 26 wherein the modification of the nucleic acid segment overexpresses the ion channel protein in the population of somatic cells.

28. The method of claim 25 or 26 wherein the modification of the nucleic acid segment produces a dominant negative ion channel proteins.

Sub B4 > 29. A method of mimicking one or more effects of a drug candidate compound in an identified somatic cell, tissue or organ of interest, the method comprising:

- a) modulating, by somatic gene transfer, expression of a selected protein in selected cells; and
- b) analyzing the result of expression of the protein to thereby predict the effect of the drug candidate compound.

30. The method of claim 29, wherein the drug candidate compound would increase or suppress activity of the protein in the cell, tissue or organ.

Sub B5 > 31. The method of claim 30, wherein the method further comprises use of a standard drug discovery strategy and use of the protein identified by the method.